

Pivotal Phase III Data in Polycythemia Vera Show that Jakafi® (ruxolitinib) Achieved Superior Disease Control Compared to Best Available Therapies

June 3, 2014

- *The trial met the primary composite endpoint of hematocrit control and at least a 35 percent reduction in spleen volume*
- *77 percent of patients treated with ruxolitinib versus 20 percent on best available therapy achieved one or both of the components of the primary endpoint*
- *Approximately half of patients in the ruxolitinib group had at least a 50 percent improvement in symptom score, compared with 5 percent on best available therapy*
- *Global regulatory filings are underway based on these data; if approved, ruxolitinib would be the first JAK1/JAK2 inhibitor available for PV patients*

Incyte to host a webcast for investors featuring key results from this trial on Tuesday, June 3, at 1:15 p.m. CDT

CHICAGO--(BUSINESS WIRE)--Jun. 3, 2014-- Incyte Corporation (Nasdaq: INCY) today announced results from the RESPONSE trial, the first pivotal Phase III study evaluating a JAK1/JAK2 inhibitor for the treatment of polycythemia vera (PV). Ruxolitinib, compared to best available therapy (BAT), significantly improved hematocrit control (red blood cell volume) without the need for phlebotomy (a procedure to remove blood from the body to reduce the concentration of red blood cells) and reduced spleen size in patients with uncontrolled PV -- those who are resistant to or intolerant of hydroxyurea (HU). Findings from the RESPONSE study are being presented in an oral presentation at the 50th Annual Meeting of the American Society of Clinical Oncology in Chicago.

"Patients with advanced PV whose disease is not well-managed with existing therapies are at increased risk for thrombosis and suffer from multiple debilitating symptoms," stated Srdan Verstovsek, M.D., Ph.D., Professor, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center. "Data from the RESPONSE trial demonstrated that treatment with ruxolitinib can consistently control hematocrit, reduce spleen size, and improve symptoms such as fatigue and itching. Importantly, there appears to be a lower rate of thrombosis in the ruxolitinib arm compared to best available therapy."

Seventy-seven percent of ruxolitinib-treated patients versus 20 percent on BAT achieved at least one component of the primary endpoint: hematocrit control from week 8 to 32 and/or at least a 35 percent reduction in spleen volume. A greater proportion of patients treated with ruxolitinib achieved the composite primary endpoint compared to BAT (21 percent vs 1 percent, respectively; $P < .0001$); 91 percent of patients in the ruxolitinib group achieving this endpoint maintained their response at week 48.

A greater proportion of patients in the ruxolitinib treatment arm had complete hematologic remission, a key secondary endpoint, when compared to the BAT arm (24 percent compared to 9 percent, $P = .003$). Patients treated with ruxolitinib also experienced meaningful improvements in PV-related symptoms: 49 percent, compared to 5 percent treated with BAT, had a 50 percent or greater improvement in symptom score at week 32 as measured by the 14-item MPN-SAF (Myeloproliferative Neoplasm Symptom Assessment Form). At week 32, one patient in the ruxolitinib group and six in the BAT group had a thromboembolic event.

At a median follow-up of 81 weeks, 85 percent of patients in the ruxolitinib arm were still receiving treatment. Because most patients in the BAT group crossed over to receive ruxolitinib therapy at week 32, adverse events were evaluated at this time when exposure between groups was similar. The most common non-hematologic adverse events of any grade in the ruxolitinib group compared to the BAT group were headache (16.4 percent vs 18.9 percent), diarrhea (14.5 percent vs 7.2 percent), fatigue (14.5 percent vs 15.3 percent), and pruritus (13.6 percent vs 22.5 percent). Based on laboratory assessments, the rates of new or worsening grade 3 or 4 anemia and thrombocytopenia in the ruxolitinib group versus the BAT group were 1.8 percent vs 0 percent and 5.5 percent vs 3.6 percent, respectively.

"One out of four patients with polycythemia vera remain uncontrolled, face a profound symptom burden and are at greater risk of cardiovascular complications such as stroke and heart attack," stated Hervé Hoppenot, President and Chief Executive Officer, Incyte. "These Phase III data give us confidence that ruxolitinib has the potential to become an important new treatment option for patients with uncontrolled PV who are no longer responding to or are intolerant of hydroxyurea."

These data will support global regulatory filings anticipated this year, including a submission to the U.S. Food and Drug Administration expected this month.

The slides used during the presentation can be accessed at [2014 ASCO - RESPONSE Presentation](#).

About the RESPONSE Trial

RESPONSE is an open-label randomized trial of 222 patients conducted in North America, Europe, Asia, and Australia. Patients with splenomegaly who were resistant to or intolerant of hydroxyurea (HU) and required phlebotomy were randomized 1:1 to receive ruxolitinib 10 mg twice daily or BAT, which was defined as investigator selected monotherapy or observation only. From week 32, patients in the BAT group could cross over to receive ruxolitinib therapy.

The primary endpoint of the study is the proportion of patients who achieved both hematocrit control without the need for phlebotomy from week 8 through 32 and a spleen volume reduction of at least 35 percent from baseline at 32 weeks. Key secondary endpoints include durable primary

response and complete hematologic remission. Complete hematologic remission was defined as maintaining hematocrit control without the need for phlebotomy, a platelet count $\leq 400 \times 10^9/L$ and white blood cell count $\leq 10 \times 10^9/L$. Other secondary endpoints include safety, symptom improvement, and quality of life.

About Polycythemia Vera

Polycythemia vera (PV) is a myeloproliferative neoplasm (MPN) characterized by an overproduction of normal red blood cells, white blood cells and platelets that leads to an increased risk of thrombosis.¹⁻⁴ Erythrocytosis (elevated red blood cell mass) is the most prominent clinical manifestation of PV, distinguishing it from other MPNs.⁵ PV may occur at any age but often presents later in life, with a median age at diagnosis of 60 years.^{6,7} Approximately 100,000 patients in the United States are living with PV,⁸ and approximately 25 percent of patients with PV develop resistance to or intolerance of hydroxyurea^{9,10} and are considered uncontrolled.

Although patients may be asymptomatic for many years, PV is associated with significant symptom burden, and the most common signs and symptoms of PV are fatigue, pruritus, night sweats, bone pain, fever and weight loss.¹¹ Splenomegaly is present in 30 percent to 40 percent of patients with PV.¹¹ In patients who experience severe and burdensome symptoms, data show that the disease causes a significant and clinically meaningful erosion of quality of life.^{12,13}

About Jakafi® (ruxolitinib)

Jakafi is a prescription medicine approved by the U.S. Food and Drug Administration to treat people with intermediate or high-risk myelofibrosis (MF), including primary MF, post-polycythemia vera MF and post-essential thrombocythemia MF. Jakafi is marketed by Incyte in the United States and by Novartis as Jakavi® (ruxolitinib) outside the United States.

Important Safety Information

Jakafi can cause serious side effects including:

Low blood counts: Jakafi may cause your platelet, red blood cell, or white blood cell counts to be lowered. If you develop bleeding, stop taking Jakafi and call your healthcare provider. Your healthcare provider will perform blood tests to check your blood counts before you start Jakafi and regularly during your treatment. Your healthcare provider may change your dose of Jakafi or stop your treatment based on the results of your blood tests. Tell your healthcare provider right away if you experience unusual bleeding, bruising, fatigue, shortness of breath, or a fever.

Infection: You may be at risk for developing a serious infection while taking Jakafi. Tell your healthcare provider if you develop symptoms such as chills, nausea, vomiting, aches, weakness, fever, or painful skin rash or blisters.

The most common side effects of Jakafi include dizziness and headache.

These are not all the possible side effects of Jakafi. Ask your healthcare provider or pharmacist for more information. Tell your healthcare provider about any side effect that bothers you or that does not go away.

Before taking Jakafi, tell your healthcare provider about all the medications, vitamins, and herbal supplements you are taking and all your medical conditions, including if you have an infection, have or had liver or kidney problems, are on dialysis, or have any other medical condition. Do not drink grapefruit juice while taking Jakafi.

Women should not take Jakafi while pregnant or planning to become pregnant, or if breast-feeding.

Please see the Full Prescribing Information available at www.jakafi.com, which includes a more complete discussion of the risks associated with Jakafi.

About the Webcast

Incyte will host an investor meeting, which will be webcast live at 1:15 p.m. CDT on June 3, 2014, and can be accessed at www.incyte.com under Investor Relations, Events and Webcasts. A replay of the event will be available for 60 days.

About Incyte

Incyte Corporation is a Wilmington, Delaware-based biopharmaceutical company focused on the discovery, development and commercialization of proprietary small molecule drugs, primarily in oncology. For additional information on Incyte, please visit the Company's website at www.incyte.com.

Forward-Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including without limitation statements with respect to the potential efficacy, safety and therapeutic value of, and Incyte's plans for, ruxolitinib in polycythemia vera, including the potential for ruxolitinib to become an important new treatment option for patients with uncontrolled polycythemia vera and the expectation to file a regulatory submission with the FDA this month, contain predictions and estimates and are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. These forward-looking statements are based on Incyte's current expectations and subject to risks and uncertainties that may cause actual results to differ materially, including unanticipated developments in and risks related to the efficacy or safety of ruxolitinib, the results of further research and development, the high degree of risk and uncertainty associated with drug development, clinical trials and regulatory approval processes, other market or economic factors, competitive and technological advances, and other risks detailed from time to time in Incyte's filings with the Securities and Exchange Commission, including its Quarterly Report on Form 10-Q for the quarter ended March 31, 2014. Incyte disclaims any intent or obligation to update these forward-looking statements.

References

1. Vannucchi AM, Guglielmelli P, Tefferi A. *CA Cancer J Clin.* 2009;59:171-91.
2. Marchioli R, Finazzi G, Specchia G, et al. *N Engl J Med.* 2013;368:22-33.

3. Tefferi A. *Am J Hematol*. 2013;88:507-16.
4. Spivak JL. *Blood*. 2002;100:4272-90.
5. Spivak JL. *Ann Intern Med*. 2010;152:300-6.
6. Tefferi A, Rumi E, Finazzi G, et al. *Leukemia*. 2013;27:1874-81.
7. Gruppo Italiano Studio Policitemia. *Ann Intern Med*. 1995;123:656-64.
8. Data on file. Incyte Corporation
9. Barosi G, Birgegard G, Finazzi G, et al. *Br J Haematol*. 2010;149:961-3.
10. Alvarez-Larrán A, Pereira A, Cervantes F, et al. *Blood*. 2012;119:1363-9.
11. Passamonti F. *Blood*. 2012;120(2):275-84.
12. Mesa RA, Niblack J, Wadleigh M, et al. *Cancer*. 2007;109(1):68-76.
13. Johansson P, Mesa R, Scherber R, et al. *Leuk Lymphoma*. 2012;53(3):441-4.



Source: Incyte Corporation

Incyte Corporation

Pamela M. Murphy

Vice President, Investor Relations & Corporate Communications

+1 302-498-6944